# Generation of expandable, self-renewing muscle stem cells for Duchenne Muscular Dystrophy

## Grant Award Details

Generation of expandable, self-renewing muscle stem cells for Duchenne Muscular Dystrophy

**Grant Type:** Inception - Discovery Stage Research Projects

**Grant Number:** DISC1-09999

**Project Objective:** The objective is to develop protocols for generating expandable, self-renewing human muscle stem cells (MuSC) from hiPS cells for Duchenne Muscular Dystrophy disease modeling and therapeutics.

**Investigator:**

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| **Name:** | Alessandra Sacco |
| **Institution:** | Sanford-Burnham Medical Research Institute |
| **Type:** | PI |

**Disease Focus:** Muscular Dystrophy

**Human Stem Cell Use:** Embryonic Stem Cell, iPS Cell

**Award Value:** $252,000

**Status:** Active

## Grant Application Details

**Application Title:** Generation of expandable, self-renewing muscle stem cells for Duchenne Muscular Dystrophy

**Public Abstract:**

**Research Objective**

The goal of this proposal is to define protocols to generate expandable, self-renewing human muscle stem cells (MuSC) from hiPS cells for Duchenne Muscular Dystrophy disease modeling and therapeutics.

**Impact**

The integration of STAT3i with current approaches to derive myogenic cells from hiPS cells would enable the generation of self-renewing MuSC that are expandable for disease modeling and therapeutics.

**Major Proposed Activities**

- Selection of the most potent STAT3i among three drugs extensively tested in preclinical and clinical studies.
- Immunophenotyping of healthy and DMD hiPS-derived MuSC treated with STAT3i to assess cell identity.
- Clonal analysis of healthy and DMD hiPS-derived MuSC treated with STAT3i to assess the composition of derived culture.
- Gene expression profiling by RNAseq to compare healthy and DMD hiPS-derived MuSC treated with STAT3i with human MuSC isolated from patients.
- Transplantation of healthy and DMD hiPS-derived MuSC in vivo into immunodeficient dystrophic mice.

**Statement of Benefit to California:**

Duchenne Muscular Dystrophy (DMD) is a lethal muscle wasting disease that affects 1:3500 males. This devastating disease is characterized by progressive loss of skeletal muscle and heart function. DMD affects more than 1,000 boys in California and poses a large economic burden for patients' families and society. Approaches to generate expandable human MuSC would be invaluable for the development of reliable disease modeling platforms as well as stem cell therapies to rescue disease progression.

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